PTC THERAPEUTICS, INC.
Form 10-Q
May 05, 2015
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

x QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the quarterly period ended March 31, 2015

OR

o TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from to

Commission file number: 001-35969

PTC Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification Number)
100 Corporate Court South Plainfield, NJ	
(Address of principal executive offices)	**07080** (Zip Code)

(908) 222-7000

(Registrant s telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes x No o

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No o

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of large accelerated filer , accelerated filer and smaller reporting company in Rule 12b-2 of the Exchange Act.

Large accelerated filer o	Accelerated filer x
Non-accelerated filer o (Do not check if a smaller reporting company)	Smaller reporting company o

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes o No x

As of April 30, 2015 there were 33,848,276 shares of Common Stock, $0.001 par value per share, outstanding.

Table of Contents

TABLE OF CONTENTS

PART I FINANCIAL INFORMATION

PART II OTHER INFORMATION

Table of Contents

FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form10-Q contains forward looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form10-Q, including statements regarding our strategy, future operations, future financial position, future revenues, projected costs, prospects, plans and objectives of management, are forward looking statements. The words anticipate, believe, estimate, expect, intend, may, might, plan, predict, project, target, potential, should, continue, and similar expressions are intended to identify forward looking statements, although not all forward looking statements contain these identifying words.

The forward looking statements in this Quarterly Report on Form 10-Q include, among other things, statements about:

- the timing and conduct of our clinical trials and studies of Translarna (ataluren) for the treatment of Duchenne muscular dystrophy, cystic fibrosis, mucopolysaccharidosis type I, or MPS I, and aniridia, caused by nonsense mutations, as well as our studies in spinal muscular atrophy and our cancer stem cell program, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials will become available;

- the rate and degree of market acceptance and clinical utility of Translarna;

- our ability to commercialize Translarna in general, and specifically as a treatment for Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, including the timing of such commercialization and our ability to successfully negotiate adequate pricing and reimbursement processes on a timely basis, or at all, in the countries in which we may obtain regulatory approval, including the countries in the European Economic Area;

- the timing of and our ability to obtain additional marketing authorization of Translarna and our other product candidates, and the ability of Translarna and our other product candidates to meet existing or future regulatory standards;

- our ability to obtain additional and maintain existing reimbursed named patient and cohort early access programs on adequate terms;

- our estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and our ability to identify such patients;

- our ability to expand the approved product label of Translarna for the treatment of nmDMD;

- the timing and scope of our commercial infrastructure expansion, including the growth of our international presence in Europe and in other territories;

- the potential receipt of revenues from future sales of Translarna and other product candidates, including our ability to earn a profit from sales or licenses of Translarna for the treatment of nmDMD;

- our sales, marketing and distribution capabilities and strategy, including the ability of our third party manufacturers to manufacture and deliver Translarna in commercially sufficient quantities and the ability of distributors to process orders in a timely manner and satisfy its other obligations to us;

- our ability to establish and maintain arrangements for the manufacture of Translarna and our other product candidates that are sufficient to meet clinical trial and commercial launch requirements;

- our plans to pursue development of Translarna for additional indications other than Duchenne muscular dystrophy, cystic fibrosis, MPS I, and aniridia, caused by nonsense mutations;

Table of Contents

- our ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area, which is conditioned upon completion of our Phase 3 confirmatory trial in nmDMD, among other things, and subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization;

- our ability to advance our earlier stage programs, including our antibacterial program;

- our plans to pursue research and development of other product candidates;

- the potential advantages of Translarna;

- our estimates regarding expenses, future revenues, capital requirements and needs for additional financing, including our ability to maintain the level of our expenses consistent with our internal budgets and forecasts and to secure additional funds on favorable terms or at all;

- our intellectual property position;

- the impact of government laws and regulations;

- our competitive position; and

- our expectations with respect to the development and regulatory status of our product candidate and program directed against spinal muscular atrophy in collaboration with F. Hoffmann La Roche Ltd and Hoffmann La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or the SMA Foundation, and our estimates regarding future revenues from achievement of milestones in that program.

We may not actually achieve the plans, intentions or expectations disclosed in our forward looking statements, and you should not place undue reliance on our forward looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in Part II, Item 1A. Risk Factors that we believe could cause actual results or events to differ materially from the forward looking statements that we make. Our forward looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2014 completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward looking statements whether as a result of new information, future events or otherwise, except as required by applicable law.

In this Quarterly Report on Form 10-Q, unless otherwise stated or the context otherwise requires, references to PTC, PTC Therapeutics, we, us, our, and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements.

PTC Therapeutics, Inc.

Consolidated Balance Sheets (unaudited)

In thousands (except per share data)

	March 31, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 36,825	$ 49,748
Marketable securities	243,642	265,493
Prepaid expenses and other current assets	6,116	3,885
Receivables, net	3,178	4,445
Total current assets	289,761	323,571
Fixed assets, net	9,408	9,159
Deposits and other assets	351	489
Total assets	$ 299,520	$ 333,219
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 23,532	$ 29,121
Deferred revenue	373	3,354
Total current liabilities	23,905	32,475
Other long-term liabilities	2,295	2,277
Total liabilities	26,200	34,752
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 33,483,306 shares at March 31, 2015. Authorized 125,000,000 shares; issued and outstanding 32,898,392 shares at December 31, 2014	33	33
Additional paid-in capital	734,489	721,722
Accumulated other comprehensive loss	(736)	(737)
Accumulated deficit	(460,466)	(422,551)
Total stockholders equity	273,320	298,467
Total liabilities and stockholders equity	$ 299,520	$ 333,219

See accompanying unaudited notes.

Table of Contents

PTC Therapeutics, Inc.

Consolidated Statements of Operations (unaudited)

In thousands (except per share data)

	Three Months Ended March 31,	
	2015	**2014**
Revenues:		
Net product revenue	$ 5,069	$
Collaboration revenue	338	9,147
Grant revenue	2,075	70
Total revenues	7,482	9,217
Operating expenses:		
Research and development	27,938	15,889
Selling, general and administrative	17,615	7,540
Total operating expenses	45,553	23,429
Loss from operations	(38,071)	(14,212)
Interest income	524	171
Other (expense) income, net	(368)	(57)
Net loss	$ (37,915)	$ (14,098)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	33,067,752	24,492,487
Net loss per share basic and diluted (in dollars per share)	$ (1.15)	$ (0.58)

See accompanying unaudited notes.

Table of Contents

PTC Therapeutics, Inc.

Consolidated Statements of Comprehensive Loss (unaudited)

In thousands

	Three Months Ended March 31,	
	2015	**2014**
Net loss	$ (37,915)	$ (14,098)
Other comprehensive loss:		
Unrealized gain on marketable securities	125	10
Foreign currency translation loss	(124)	
Comprehensive loss	$ (37,914)	$ (14,088)

See accompanying unaudited notes.

Table of Contents

PTC Therapeutics, Inc.

Consolidated Statements of Cash Flows (unaudited)

In thousands

	Three months ended March 31,	
	2015	**2014**
Cash flows from operating activities		
Net loss	$ (37,915)	$ (14,098)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	634	588
Change in valuation of warrant liability	41	55
Amortization of premiums on investments	486	414
Share-based compensation expense	9,748	3,705
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	(2,231)	(358)
Receivables	1,267	120
Deposits and other assets	138	17
Accounts payable and accrued expenses	(5,589)	(3,472)
Other long-term liabilities	(23)	(23)
Deferred revenue	(2,981)	(386)
Net cash used in operating activities	(36,425)	(13,438)
Cash flows from investing activities		
Purchases of fixed assets	(883)	(186)
Purchases of marketable securities	(19,108)	(25,354)
Sale & redemption of marketable securities	40,598	21,273
Net cash provided/(used in) investing activities	20,607	(4,267)
Cash flows from financing activities		
Payments on long-term debt		(37)
Proceeds from exercise of options	3,019	
Net proceeds from public offerings		118,183
Net cash provided by financing activities	3,019	118,146
Effect of exchange rate changes on cash	(124)	
Net increase/(decrease) in cash and cash equivalents	(12,923)	100,441
Cash and cash equivalents, beginning of period	49,748	15,414
Cash and cash equivalents, end of period	$ 36,825	$ 115,855
Supplemental disclosure of cash information		
Cash paid for interest	$	$ 1
Supplemental disclosures of non-cash information related to investing and financing activities		
Change in unrealized gain (loss) on marketable securities	$ 125	$ 10

See accompanying unaudited notes.

Table of Contents

PTC Therapeutics, Inc.

Notes to Consolidated Financial Statements (unaudited)

March 31, 2015

In thousands (except per share data unless otherwise noted)

1. The Company

PTC Therapeutics, Inc. (the Company or PTC) was incorporated as a Delaware corporation on March 31, 1998. PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, small molecule therapeutics targeting an area of RNA biology referred to as post-transcriptional control. The letters PTC in the corporate name are an acronym for post-transcriptional control processes, which are the regulatory events that occur in cells during and after a messenger RNA is copied from DNA through the transcription process. The Company has discovered all of its compounds currently under development using its proprietary technologies. The Company plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. The Company believes that systematically targeting post-transcriptional control processes represents an unexploited approach to drug discovery and development. The Company s internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases.

The Company s lead product, Translarna (ataluren) received marketing authorization from the European Commission, or EC, in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy, or nmDMD, in ambulatory patients age 5 years and over in the 31 member states of the European Economic Area, or EEA. This marketing authorization is subject to annual review and renewal by the European Medicines Agency, or EMA following its reassessment of the risk-benefit balance of the authorization and is further conditioned on the Company s ability to complete its global, confirmatory Phase 3 clinical trial in nmDMD, which it refers to as ACT DMD, and submit the final report, including additional efficacy and safety data from the trial, during 2015. See Risk Factors Risks Related to Regulatory Approval of our Product Candidates for further detail regarding the EMA s approval process, including a description of the risk-benefit balance.

The Company launched Translarna on a commercial basis in Germany in December 2014 and expects to expand its launch activities across the EEA throughout 2015 and future years, subject to successful completion of pricing and reimbursement negotiations. Concurrently, the Company has been pursuing reimbursed early access programs in selected countries where those mechanisms exist, both within Europe and in those countries outside of Europe that will reference the marketing authorization described above.

The Company has not generated significant product revenue to date and is subject to a number of risks similar to those of other early stage companies, including dependence on key individuals, the difficulties inherent in the development of commercially usable products, the potential need to obtain additional capital necessary to fund the development of its products, and competition from other companies. As of March 31, 2015, the Company had an accumulated deficit of approximately $460.4 million. The Company has financed its operations to date primarily through public offerings of common stock in February 2014 and October 2014, its initial public offering of common stock in June 2013, private placements of its convertible preferred stock, collaborations, bank debt, convertible debt financings, grant funding and clinical trial support from governmental and philanthropic organizations and patient advocacy groups in the disease area addressed by the Company s product candidates.

2. Summary of significant accounting policies

The Company s complete listing of significant accounting policies are described in Note 2 of the notes to the Company s audited financial statements as of December 31, 2014 included in the Company s Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 2, 2015 (2014 Form 10-K).

Basis of Presentation

The accompanying financial information as of March 31, 2015 and for the three months ended March 31, 2015 and 2014 has been prepared by the Company, without audit, pursuant to the rules and regulations of the SEC. Certain information and footnote disclosures normally included in financial statements prepared in accordance with

Table of Contents

generally accepted accounting principles in the United States (GAAP) have been condensed or omitted pursuant to such rules and regulations. These interim financial statements should be read in conjunction with the Company s audited financial statements as of December 31, 2014 and notes thereto included in the 2014 Form 10-K.

In the opinion of management, the unaudited financial information as of March 31, 2015 and for the three months ended March 31, 2015 and 2014 reflects all adjustments, which are normal recurring adjustments, necessary to present a fair statement of financial position, results of operations and cash flows. The results of operations for the three month period ended March 31, 2015 are not necessarily indicative of the results to be expected for the year ended December 31, 2015 or for any other interim period or for any other future year.

Use of estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements, and the reported amounts of revenues and expenses during the reporting period. Actual results could differ from those estimates.

Inventories and cost of product revenue

On August 4, 2014, the Company was notified that the European Commission, or EC, granted marketing authorization for Translarna for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, in ambulatory patients aged five years and older. The marketing authorization allows the Company to market Translarna in the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland and Liechtenstein. This marketing authorization is subject to annual review and renewal by the European Medicines Agency, or EMA following its reassessment of the risk-benefit balance of the authorization and is further conditioned on the Company s ability to complete its global, confirmatory Phase 3 clinical trial in nm DMD, which it refers to as ACT DMD, and submit the final report, including additional efficacy and safety data from the trial during 2015. The launch in these countries is on a country by country basis. The Company does do not have sufficient history or experience from which to accurately forecast product sales or demand generation. As such, the Company has not capitalized inventory and will not capitalize inventory until the completion of ACT DMD and satisfaction of the EMA conditions or until the Company can reasonably predict future product sales. The costs incurred related to the manufacturing of Translarna have been recorded as research and development expense in the statements of operations. The Company s cost of product sales includes royalties and other miscellaneous selling costs, which were not material and therefore included as a component of research and development costs in the current year presentation. The time period over which this inventory is consumed will depend on a number of factors, including the amount of future Translarna sales, and the ability to utilize inventory prior to its expiration date.

Recently issued accounting standard

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2014-09, Revenue from Contracts with Customers. ASU 2014-09 will eliminate transaction- and industry-specific revenue recognition guidance under current GAAP and replace it with a principle-based approach for determining revenue recognition. ASU 2014-09 will require that companies recognize revenue based on the value of transferred goods or services as they occur in the contract. The ASU also will require additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes

in judgments and assets recognized from costs incurred to obtain or fulfill a contract. ASU 2014-09 is effective for annual reporting periods beginning after December 15, 2016. Early application is not permitted. Entities can transition to the standard either retrospectively or as a cumulative-effect adjustment as of the date of adoption. Presently, the Company is assessing what effect the adoption of ASU 2014-09 will have on its financial statements and accompanying notes.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements Going Concern Disclosures of Uncertainties about an entity s Ability to Continue as a Going Concern. ASU 2014-15 provides new guidance related to management s responsibility to evaluate whether there is substantial doubt about an entity s ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in

Table of Contents

U.S. auditing standards and to provide related footnote disclosures. This new guidance is effective for the annual period ending after December 15, 2016, and for annual periods and interim periods thereafter. The requirements of ASU 2014-15 are not expected to have a significant impact on the Condensed Consolidated Financial Statements.

Revenue Recognition

The Company recognizes revenue when amounts are realized or realizable and earned. Revenue is considered realizable and earned when the following criteria are met: (1) persuasive evidence of an arrangement exists; (2) delivery has occurred or services have been rendered; (3) the price is fixed or determinable; and (4) collection of the amounts due are reasonably assured.

Net Product Sales

PTC S net product sales have consisted solely of sales of Translarna for the treatment of nmDMD in territories outside of the U.S. The Company applies the revenue recognition guidance in accordance with Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) Subtopic 605-15, Revenue Recognition Products. The Company recognizes revenue from product sales when there is persuasive evidence that an arrangement exists, title to product and associated risk of loss has passed to the customer, the price is fixed or determinable, collectability is reasonably assured and the Company has no further performance obligations.

The Company records revenue on sales where Translarna is available either on a commercial basis or through a reimbursed early access program and typically paid for by a government authority or institution. Prior to January 1, 2015, the Company recognized revenue for commercial and reimbursed early access program sales on a cash basis once the product was shipped on behalf of the government authority or institution and payment had been received, if all other revenue recognition criteria were met. Beginning in the first quarter of 2015, the Company is recognizing revenue for Translarna as product is shipped, as the Company has established a pattern of collectability.

The Company records revenue net of estimated discounts and rebates. Allowances are recorded as a reduction of revenue at the time revenues from product sales are recognized. Allowances for government rebates and discounts are established at the time of delivery. These allowances are adjusted to reflect known changes in factors that may impact such allowances in the quarter those changes are known.

Collaboration and Grant Revenue

The terms of these agreements typically include payments to the Company of one or more of the following: nonrefundable, upfront license fees; milestone payments; research funding and royalties on future product sales. In addition, the Company generates service revenue through agreements that generally provide for fees for research and development services and may include additional payments upon achievement of specified events.

The Company evaluates all contingent consideration earned, such as a milestone payment, using the criteria as provided by the Financial Accounting Standards Board (FASB), guidance on the milestone method of revenue recognition. At the inception of a collaboration arrangement, the Company evaluates if a milestone payment is substantive. The criteria requires that (1) the Company determines if the milestone is commensurate with either its performance to achieve the milestone or the enhancement of value resulting from our activities to achieve the milestone; (2) the milestone be related to past performance; and (3) the milestone be reasonable relative to all deliverable and payment terms of the collaboration arrangement. If these criteria are met then the contingent milestones can be considered a substantive milestone and will be recognized as revenue in the period that the milestone is achieved. The Company recognizes royalties as earned in accordance with the terms of various research and collaboration agreements. If not substantive, the contingent consideration is allocated to the existing units of accounting based on relative selling price and recognized following the same basis previously established for the associated unit of accounting.

The Company recognizes revenue for reimbursements of research and development costs under collaboration agreements as the services are performed. The Company records these reimbursements as revenue and not as a reduction of research and development expenses as the Company has the risks and rewards as the principal in the research and development activities.

Table of Contents

3. Fair value of financial instruments and marketable securities

The Company follows the fair value measurement rules, which provides guidance on the use of fair value in accounting and disclosure for assets and liabilities when such accounting and disclosure is called for by other accounting literature. These rules establish a fair value hierarchy for inputs to be used to measure fair value of financial assets and liabilities. This hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three levels: Level 1 (highest priority), Level 2, and Level 3 (lowest priority).

- Level 1 Unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the balance sheet date.

- Level 2 Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly. Level 2 inputs include quoted prices for similar assets and liabilities in active markets, quoted prices for identical or similar assets or liabilities in markets that are not active, inputs other than quoted prices that are observable for the asset or liability (i.e., interest rates, yield curves, etc.), and inputs that are derived principally from or corroborated by observable market data by correlation or other means (market corroborated inputs).

- Level 3 Inputs are unobservable and reflect the Company s assumptions as to what market participants would use in pricing the asset or liability. The Company develops these inputs based on the best information available.

Cash equivalents are reflected in the accompanying financial statements at fair value. The carrying amount of grant and collaboration receivables, accounts payable and accrued expenses, and debt approximates fair value due to the short-term nature of those instruments.

Fair value of certain marketable securities is based upon market prices using quoted prices in active markets for identical assets quoted on the last day of the period. In establishing the estimated fair value of the remaining investments, the Company used the fair value as determined by its investment advisors using observable inputs other than quoted prices.

The Company reviews its investments on a periodic basis for other-than-temporary impairments. This review is subjective, as it requires management to evaluate whether an event or change in circumstances has occurred in that period that may have a significant adverse effect on the fair value of the investment.

Table of Contents

The following represents the fair value using the hierarchy described in Note 3 for the Company s financial assets and liabilities that are required to be measured at fair value on a recurring basis as of March 31, 2015 and December 31, 2014:

	March 31, 2015			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 243,642	$	$ 243,642	$
Warrant liability	229			229

	December 31, 2014			
	Total	Quoted prices in active markets for identical assets (level 1)	Significant other observable inputs (level 2)	Significant unobservable inputs (level 3)
Marketable securities	$ 265,493	$	$ 265,493	$
Warrant Liability	188			188

The following is a summary of marketable securities accounted for as available-for-sale securities at March 31, 2015 and December 31, 2014:

	March 31, 2015			
	Amortized Cost	Gross Unrealized Gains	Losses	Fair Value
Commercial paper	$ 19,748	$ 26	$	$ 19,774
Corporate debt securities	186,810	44		